Proof of concept studies exploring the safety and functional activity of human parthenogenetic-derived neural stem cells for the treatment of Parkinson's disease.

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Public Summary:
Recent studies indicate that human pluripotent stem cell (PSC)-based therapies hold great promise in Parkinson's disease (PD). Clinical studies have shown that grafted fetal neural tissue can achieve considerable biochemical and clinical improvements in PD. However, the source of fetal tissue grafts is limited and ethically controversial. Human parthenogenetic stem cells offer a good alternative because they are derived from unfertilized oocytes without destroying viable human embryos and can be used to generate an unlimited supply of neural stem cells for transplantation. Here we evaluate for the first time the safety and engraftment of human parthenogenetic stem cell-derived neural stem cells (hpNSCs) one rodent and one non-human primate model of PD. In both rodents and nonhuman primates, we observed successful engraftment and higher dopamine levels in hpNSC-transplanted animals compared to vehicle control animals, without any adverse events. These results indicate that hpNSCs are safe, well tolerated, and could potentially be a source for cell-based therapies in PD.

Scientific Abstract:
Recent studies indicate that human pluripotent stem cell (PSC)-based therapies hold great promise in Parkinson's disease (PD). Clinical studies have shown that grafted fetal neural tissue can achieve considerable biochemical and clinical improvements in PD. However, the source of fetal tissue grafts is limited and ethically controversial. Human parthenogenetic stem cells offer a good alternative because they are derived from unfertilized oocytes without destroying viable human embryos and can be used to generate an unlimited supply of neural stem cells for transplantation. Here we evaluate for the first time the safety and engraftment of human parthenogenetic stem cell-derived neural stem cells (hpNSCs) in two animal models: 6-hydroxydopamine (6-OHDA)-lesioned rodents and 1-methyl-4-phenyl-1,2,3,6-tetrahydropyridine (MPTP)-treated nonhuman primates (NHPs). In both rodents and nonhuman primates, we observed successful engraftment and higher dopamine levels in hpNSC-transplanted animals compared to vehicle control animals, without any adverse events. These results indicate that hpNSCs are safe, well tolerated, and could potentially be a source for cell-based therapies in PD.